NPLB No Patient Left Behind

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Kathleen Cantwell, Director
CMS Office of Strategic Operations and Regulatory Affairs
Division of Regulations Development
Attention: Document Identifier CMS–10844
Room C4–26–05
7500 Security Boulevard
Baltimore, Maryland 21244–1850

Dear Director Cantwell,

Thank you for the opportunity to comment on the Inflation Reduction Act's (IRA) "Small Biotech Exception" Information Collection Request (ICR).

I am writing on behalf of No Patient Left Behind (NPLB), a non-profit organization comprised of biotech investors, innovators, researchers, physicians, and patient advocates dedicated to three guiding principles: 1) prescribed treatments authorized by an insurer should be affordable to patients through zero or low out-of-pocket costs; 2) drug prices should not stay high for too long; and 3) sufficient R&D incentives must remain in place to ensure continued U.S. biotech innovation.

Unfortunately, NPLB does not believe the IRA's "exceptions" to protect biotech small business, orphan drug innovators, or less-successful commercial innovations will work as intended as detailed on the next page. We believe the best way to fix IRA's ineffective exceptions is to have the government treat small and large molecules the same and set their Medicare negotiated price at 13 years after FDA approval. Such a fix would ensure adequate protections as the bill intended without ineffective special carve-outs. It also would ensure patients and society equally benefit by ensuring small molecules are not disincentivized in favor of large molecule drugs for diseases of the aging.

NPLB agreed with the intent of IRA and would have supported the law's passage had it included two important modifications: 1) treat small & large molecules equally by allowing the government to set prices 13 years after FDA approval, and 2) require Medicare Advantage plans to cap beneficiary catastrophic prescription drug out-of-pocket costs at roughly $1500 per year to ensure at least 1/3rd of non-Low Income Subsidy (LIS) seniors benefitted from the law's catastrophic insurance reforms.

As CMS undertakes the challenging task to implement IRA, NPLB remains deeply concerned that IRA's discrimination against small molecule innovations for diseases of the aging will have lasting negative consequences for patients and society. Prior to the bill's passage our members shared this investor analysis with policymakers about the impact the legislation would have on new small molecule R&D. Our members also have written how the bill will increase societal costs over time by incentivizing pills over shots that, while just as risky to innovate, large molecules are harder to genericize, often require physician administration, and often are reimbursed under Part B for which the IRA does not provide new catastrophic coverage. NPLB even proposed deeper minimum government discounts at year 13 as a revenue neutral solution to ensure the bill did not discriminate against small molecule R&D for diseases of the aging.

NPLB continues to provide research, data, & education about the legislation's real-world impact on drug affordability and innovation -- including this recent presentation on how IRA has made new small molecule R&D uninvestable and this letter from biotech leaders highlighting how fixing IRA matters to all of society.

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Why IRA's "Exceptions" Do Not Work

1. ***Small biotech exception not relevant for any drugs in development today***
   The IRA only exempts small biotech companies from government negotiated price-setting from 2026-2028. Given the IRA's negotiation and price-setting timeline, even without the exemption, no small biotech company products would be eligible for government negotiated price setting until after 2030. Even if the exemption did apply to a small biotech with a government negotiated price-setting eligible product, the IRA's time-limited exception depresses the value of the company and makes it less likely to attract significant new financing or be acquired by a large manufacturer. The exception does not protect small biotechs that are acquired by larger companies and investors are less willing to fund a drug's development if it lacks the capabilities of a large manufacturer to meet commercial demand and receive adequate formulary placement amongst the highly market-concentrated vertically integrated Medicare Advantage health plans and pharmacy benefit managers (PBMs).

2. ***Orphan exception disincentives companies from pursuing multiple orphan indications***
   Drugs with multiple orphan drug designations are not exempt from the IRA's government negotiated price-setting. Most orphan drugs that are relevant for Medicare populations are oncology drugs that have orphan drug designation for multiple diseases (e.g. lung cancer and colorectal cancer with a specific genetic mutation). Rather than encourage orphan drug R&D, the provision forces companies to make the impossible choice of which one orphan indication to pursue, when they could pursue many.

3. ***Exception for limited revenue therapies***
   Any drug with fewer than $200M in total sales 9 years after FDA approval is already a commercial failure and likely did not justify the ~$2.5B of R&D costs it took to develop the therapy. While patients and society may benefit from the incremental improvement or the therapeutic bridge the less-successful innovation may have allowed until a better innovation or cure was developed, price set by Medicare, or genericized, the manufacturer still unlikely is to be materially helped by the IRA's exception.

Once again, NPLB supports IRA's intent to achieve biotech affordability and innovation. Unfortunately, while the IRA exceptions may sound rhetorically pleasing, we do not believe they will have their intended impact of protecting small biotechs, orphan disease research, or less successful products from IRA's harms. We recommend the administration take action to fulfill the intent of the exceptions by seeking positive improvements that extend the law's insurance reforms and catastrophic caps to more seniors and treats all innovative therapies fairly and equally at 13 years after FDA approval.

Sincerely,

Peter Rubin
Executive Director
No Patient Left Behind (NPLB)